

TRANSKARYOTIC PRODUCTION AND DELIVERY OF DNASE

Abstract of the Disclosure

5 The invention relates to novel human DNA sequences,
targeting constructs, and methods for producing novel genes
encoding thrombopoietin, DNase I, and β -interferon by
homologous recombination. The targeting constructs
comprise at least: a) a targeting sequence; b) a regulatory
sequence; c) an exon; and d) a splice-donor site. The
10 targeting constructs, which can undergo homologous
recombination with endogenous cellular sequences to
generate a novel gene, are introduced into cells to produce
homologously recombinant cells. The homologously
recombinant cells are then maintained under conditions
15 which will permit transcription of the novel gene and
translation of the mRNA produced, resulting in production
of either thrombopoietin, DNase I, or β -interferon. The
invention further relates to a methods of producing
pharmaceutically useful preparations containing
20 thrombopoietin, DNase I, or β -interferon from homologously
recombinant cells and methods of gene therapy comprising
administering homologously recombinant cells producing
thrombopoietin, DNase I, or β -interferon to a patient for
therapeutic purposes.

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